NIH INDI PROJECT

Industrialized CRISPR iPS Cells Enable NIH Large Scale Alzheimer's Disease Research Effort

Background & Goals

Unraveling the Genetics of Alzheimer's and Dementias

Alzheimer's disease and related dementias (ADRD) are progressive neurodegenerative disorders affecting over **6 million people in the United States**—a number projected to **nearly triple by 2050** due to an aging population. Globally, ADRD impacts more than **55 million** individuals, with a new case developing every **three seconds**. This surge presents a looming public health crisis, with annual care costs expected to reach **$1.1 trillion** in the U.S. alone by 2050.

Despite significant advances, current treatments offer only symptomatic relief without halting disease progression. Genetics plays a pivotal role in ADRD, with **over 50 genes** associated with these conditions.

Understanding the intricate interactions among these genes is crucial for:

- **Early Detection:** Identifying genetic markers for prompt diagnosis.
- **Personalized Medicine:** Developing targeted therapies based on individual genetic profiles.
- **Disease Prevention:** Unveiling genetic risk factors to inform preventive strategies.

> Unlocking these genetic complexities is essential for combating these devastating diseases and improving millions of lives.

Challenge

Scaling CRISPR Editing for Neurodegenerative Disease Research

Investigating the genetic underpinnings of ADRD demands creating a comprehensive library of genetically edited induced pluripotent stem (iPS) cell lines. These cells can be differentiated into neurons, providing human-based models to study disease mechanisms and test potential therapies.

However, generating this library poses significant challenges:

- **Complexity of Editing Multiple Genes:** Understanding gene interplay requires editing numerous genes, increasing complexity exponentially.
- **Technical Challenges with iPS Cells:** iPS cells are delicate and require stringent conditions to maintain pluripotency, making them challenging to edit and cultivate.
- **Resource Intensive:** Researchers spend an average of weeks, if not months, of hands-on time per project, often with uncertain outcomes.
- **Scaling Limitations:** Traditional CRISPR workflows are laborious and time-consuming, hindering large-scale studies.

> These obstacles create bottlenecks in ADRD research, delaying potential breakthroughs and the development of effective treatments.

Solution

EditCo and the NIH iNDI Project: Transforming CRISPR Editing at Scale

To overcome these challenges, EditCo (formerly Synthego) partnered with the National Institutes of Health (NIH) on the **iPSC Neurodegenerative Disease Initiative (iNDI)** project. The iNDI project aims to accelerate neurodegenerative disease research by creating a publicly available, comprehensive library of CRISPR-engineered iPS cell lines representing various genetic mutations associated with ADRD.

EditCo's Contributions:

- **Industrialized CRISPR Editing Platform:** Leveraging our high-throughput CRISPR editing capabilities, we efficiently generated a large number of precisely edited iPS cell lines required for the iNDI project.
- **Expertise in iPS Cell Editing:** Our proprietary protocols ensured high-efficiency gene edits while maintaining cell viability and pluripotency, crucial for downstream differentiation into neurons.
- **Quality and Precision:** Utilizing state-of-the-art reagents and rigorous quality control processes, we delivered iPS cell lines with accurate gene edits.

Empowering Breakthroughs with EditCo

EditCo's partnership with the NIH on the iNDI project exemplifies our commitment to advancing neurodegenerative disease research. By industrializing CRISPR editing, we enable researchers to:

- **Unlock Genetic Insights:** Study the complex interactions among ADRD-associated genes with unprecedented efficiency.
- **Develop Targeted Therapies:** Accelerate the identification and testing of potential therapeutic targets.
- **Improve Patient Outcomes:** Contribute to the development of treatments that could halt or reverse disease progression.

With EditCo's innovative solutions, the quest to understand and combat Alzheimer's disease and related dementias is more achievable than ever before.

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